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**A scalable approach to prevent teratoma formation of human embryonic stem cells.**

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**Public Summary:**

hESCs can undergo unlimited self-renewal and differentiate into all cell types in the body. Therefore, hESCs hold great promise for human cell therapy. One of the key bottlenecks that hinder the clinic development of hESCs is the teratoma risk of hESCs. In this report, we developed an efficient and scalable approach to eliminate the teratoma risk of hESCs and could facilitate the clinical development of hESC-based therapy.

**Scientific Abstract:**

As the renewable source of all cell types in the body, human embryonic stem cells (hESCs) hold great promise for human cell therapy. However, one major bottleneck that hinders the clinic application of hESCs is that hESCs remaining with their differentiated derivatives pose cancer risk by forming teratomas after transplantation. NANOG is a critical pluripotency factor specifically expressed in hESCs but rarely in their differentiated derivatives. By introducing a hyperactive variant of herpes simplex virus thymidine kinase gene into the 3'-untranslated region of the endogenous NANOG gene of hESCs through homologous recombination, we developed a safe and highly scalable approach to efficiently eliminate the teratoma risk associated with hESCs without apparent negative impact on their differentiated cell types. As thymidine kinase is widely used in human gene therapy trials and is the therapeutic target of U. S. Food and Drug Administration-approved drugs, our strategy could be effectively applied to the clinic development of hESC-based human cell therapy.

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